



# INTERNATIONAL E-WORKSHOP FRAGILE X & ASSOCIATED CONDITIONS MARCH 18TH & 19TH 2021 - ONLINE



In collaboration with its **Scientific Board**, and in partnership with **DéfiScience** (the National Network for Intellectual Disabilities), the **Fragile X France association** ([www.xfra.org](http://www.xfra.org)) is proud to announce its next **international workshop** dedicated to the **Fragile X Syndrome and premutation associated conditions**.

This collaborative working meeting aims to connect **researchers & clinicians** working on Fragile X syndrome in order to **consolidate or bring out new therapeutic leads**.

Given the still uncertain health context, the Workshop on Fragile X is organized online.

## PROGRAM

### THURSDAY MARCH, 18TH

#### 10 a.m. to 1 p.m. : Clinical update

Clinical genetics in 2020 (*Delphine Héron*)

Clinical follow up for Fragile X (*Vincent des Portes*)

Impact on daily life (*Fragile X France*)

Impact on daily life : DIXIT study (*Aurore Curie & Clémentine Fort*)

FXTAS (*Perrine Charles*)

FXPOI (*Anne Bachelot*)

#### 2 p.m. to 5 p.m. : Physiopathology of Fragile X Syndrome

FMRP : function, molecular targets, mouse models (*Hervé Moine*)

Neurobiology of FXTAS and therapeutic approaches (*Nicolas Charlet Berguerand*)

Neurobiology of Fragile X : revolution of iPSC and organoïdes (*Peng Jin*)

Modeling Fragile X Syndrome with iPSCs (*Cecilia Laterza*)

### FRIDAY MARCH, 19TH

#### 10 a.m. to 1 p.m. : Therapeutics of Fragile X Syndrome

What therapeutic trial plans ? (*Aurore Curie*)

Efficacy judgment criteria in Fragile X (*Aurore Curie & Perrine Charles*)

Identification of non-invasive clinical markers of FXS (*Sylvain Briault*)

Fragile X : 30 years later : satisfactions, worries, expectations (*Fragile X France*)

Fragile X : 30 years of research (*Jean-Louis Mandel - subject to change*)

#### 2 p.m. to 5 p.m. : Innovative therapies, drug candidates

Therapeutic trials stopped & in progress, what lessons ? (*Sébastien Jacquemont - subject to change*)

DGKK gene therapy for Fragile X treatment : the GETEX project (*Hervé Moine*)

Therapeutic Potential of CRISPR/Cas9 Mediated Deletion of CGG repeats for FMR1 Gene Reactivation in Fragile X Syndrome (*Daman Kumari*)

Involvement of Phosphodiesterase 2A Activity : a new treatment for Fragile X Syndrome and other forms of neuronal development disease (*Barbara Bardoni*)

#### 5 p.m. to 5:15 p.m. : Conclusion

## INFORMATIONS

### PUBLIC

Clinicians & Researchers

All discussions in English

### DATES

March 18th & 19th, 2021

From 10 a.m. to 1 p.m.

And from 2 p.m. to 5 p.m.

### REGISTRATIONS

Registration required

Possible until march 17th, 2021

[www.xfra.org](http://www.xfra.org)

[www.defiscience.fr](http://www.defiscience.fr)

### PRICES

Free registration

### CONTACT

For any further information or comments, please contact us at :

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